ROUTING SLIP
GENERATED BY: HF-40
DATE: JUL 14, 2004

FDA CONTROL NUMBER: 04 3877 **TRACER #:** **OS #:**

DATE OF CORRESPONDENCE: 07/06/04 **DATE INTO FDA:** 07/14/04

TO: LESTER M CRAWFORD, ACTING COMMISSIONER OF THE FOOD AND DRUG ADMINISTRATION

FROM: DAYTON T REARDAN, ORPHAN MEDICAL, INC.

SYNOPSIS: FORWARDS COPY OF LETTER FROM ABBEY MEYERS (NATIONAL ORGANIZATION FOR RARE DISORDERS, INC.). REQUESTS THAT LETTER BE ADDED TO PENDING CITIZEN PETITION (DOCKET # 003P-0039/CPI).

LEAD OFFICE: HFA-305 **HOME OFFICE:** HF-40

CONTACT/PHONE#: CAPRI R MCCLENDON 301-827-5903

COPIES: HF-40 ELIZABETH A CLARKE

COORDINATION:

SIGNATURE REQUIRED:

REFERRALS FROM HF-40

ASSIGNED TO	ACTION	DUE DATE
HFA-305	NECESSARY ACTION	

ORPHAN MEDICAL

July 6, 2004

Lester M. Crawford, DVM, PhD
Acting Commissioner [HF-2]
Food and Drug Administration
Room 14-71
Parklawn Building,
5600 Fishers Lane
Rockville, MD 20857
Phone: 301-827-2410

Subject: **Docket Number 03P-0039/CP1**
Request Addition of NORD Letter to Current Citizen Petition

Dear Dr. Crawford:

On January 28, 2003, our Company submitted a Citizen Petition to address serious issues that have arisen in the application of product and establishment user fees to orphan drugs. On April 22, 2003, Orphan Medical met with Dan Troy and other FDA staff regarding this Citizen Petition.

We respectfully request you add the attached May 7, 2004 letter authored by Abbey Meyers, President of the National Organization for Rare Disorders, Inc. to this pending, yet to be resolved, Citizen's Petition docket.

Please contact me directly, should you have any questions or concerns regarding this correspondence.

Sincerely yours,

Dayton T. Reardan, PhD, RAC
Vice President of Regulatory Affairs
Orphan Medical, Inc.
Direct: 952-513-6969

Enclosure

cc: Abbey Meyers, NORD
Jane Axelrad, FDA – CDER
John McCormick, FDA – OOPD
Bo Allen, Rare Disease Therapeutics

Dedicated to Patients with Uncommon diseases®
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CI 3877

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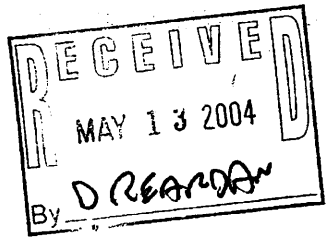
...out of the darkness, into the light...

May 7, 2004



Marlene E. Haffner, M.D.
FDA Office For Orphan Products
HF35, RM 8-73
5600 Fishers Lane
Rockville, MD 20857

Dear Marlene:

In response to Bo Allen's letter of May 3, I want to say on behalf of rare disease patients that Bo is absolutely correct; something must be done about the ludicrous user fees that FDA is charging to manufacturers of orphan products.

As you know, I attended a meeting last year with Dan Troy and Orphan Medical (Jack McCormack was there) about this same topic. In that situation, Orphan Medical had a product with one-half million dollars annual sales, and the FDA demanded user fees totaling more than a half million dollars. In that case, the woman in charge of user fees said the manufacturer has to be a small company with less than $10 million in sales; therefore, despite the ridiculous situation with this particular half million-dollar drug, Orphan Medical was not eligible for the waiver.

As you know, it was NORD's advocacy efforts that got the orphan drug user fee waiver inserted in one of the PDUFA laws. Since FDA refuses to budge on this issue, the agency will leave us no other option but to develop another amendment that will force the agency to observe the intent of the waiver. It is ludicrous that FDA uses a definition for a small business that is different than all other government agencies. I believe the definition used by the agency is a business with less than $10 million annual sales, whereas the small business administration's definition is much more liberal.

In the case of Rare Disease Therapeutics (RDT), the FDA is looking at the sales of Swedish Orphan and not the sales of RDT. RDT licenses the drug from Swedish Orphan so only RDT's annual revenues should be recognized. Similarly, FDA counts the sales of all of Orphan Medical's drugs inside and outside of the USA, and that should not be allowed because the sales of all products the company makes and sells in all countries is not related to the user fees for one orphan drug sold to a small number of people in the USA.

The bottom line is that patients are paying a huge unbearable tax on orphan drugs. Companies must pass these expenses onto patients, and the rarer a disease is the higher the tax is. As Bo points out, patients with common conditions pay just a few pennies compared to thousands of dollars tacked on to each orphan drug.

Marlene, I hope you can do something to find an amicable solution because it is prejudicial to allow this situation to continue. As patient advocates, we must do something to stop these costs from being passed onto patients. The amendment that authorized the waiver makes it clear that FDA cannot expect rare disease patients to bear these costs, whereas if you increase user fees for a drug like Celebrex or Viagara, it would hardly be noticeable (amounting to fractions of pennies per person). If we must resort to the legislative route, FDA many not like the language of a new amendment so it would be much better to resolve this user fee problem amicably.

I look forward to your reply.

Very truly yours,

Abbey S. Meyers
President

ASM:aa

cc: Bo Allen
Milton Ellis
Diane Dorman
Dr. Jack McCormack

bc: Dayton Reardon
John Ballion